

### PATENT COOPERATION TREATY

# **PCT**

### INTERNATIONAL SEARCH REPORT

(PCT Article 18 and Rules 43 and 44)

Applicant's or agent's file reference 10589-34-228	FOR FURTHER ACTION		orm PCT/ISA/220 re applicable, item 5 below.
International application No. PCT/US04/09590	International filing date (day/mo 26 March 2004 (26.03.2004)	onth/year)	(Earliest) Priority Date (day/month/year) 27 March 2003 (27.03.2003)
Applicant PTC THERAPEUTICS, INC.			
according to Article 18. A copy is being  This international search report consists	g transmitted to the International of a total of sheets.	Bureau.	thority and is transmitted to the applicant
It is also accompanied	l by a copy of each prior art doc	ument cited is	n this report.
	international search was carried onless otherwise indicated under th		is of the international application in the
	search was carried out on the bas rity (Rule 23.1(b)).	sis of a transla	tion of the international application
b. With regard to any nucleoti	de and/or amino acid sequence o	disclosed in the	e international application, see Box No. I.
2. Certain claims were found	unsearchable (See Box No. II)		
3. Unity of invention is lacking	ig (See Box No. III)		
4. With regard to the title,	itted by the applicant		
the text is approved as subm	by this Authority to read as follow		•
5. With regard to the abstract,			
the text is approved as submi	••		
			s it appears in Box No. IV. The applicant report, submit comments to this Authority.
6. With regard to the drawings,	and the body and the body and the Principles		
a. the figure of the drawings to be p  as suggested by the a	oublished with the abstract is Figure	re No	
	uthority, because the applicant fai	iled to suggest	a figure
	uthority, because this figure better		-
b. none of the figures is to be pu		i characterizes	one myenton.

Form PCT/ISA/210 (first sheet) (January 2004)

## INTERNATIONAL SEARCH REPORT

International application No.

PCT/US04/09590

IPC(7) US CL	SSIFICATION OF SUBJECT MATTER  : A01N 61/00; C12Q 1/00; G01N 33/566, 33/573, 33/574  : 435/ 4, 6, 7.2, 7.21, 41, 69.2, 91.3, 183; 514/ 1, 2  o International Patent Classification (IPC) or to both national classification and IPC	
	DS SEARCHED	
	ocumentation searched (classification system followed by classification symbols) 35/4, 6, 7.2, 7.21, 41, 69.2, 91.3, 183; 514/1, 2	
Documentati	on searched other than minimum documentation to the extent that such documents are included i	n the fields searched
	ata base consulted during the international search (name of data base and, where practicable, searontinuation Sheet	rch terms used)
	UMENTS CONSIDERED TO BE RELEVANT	
Category *	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
Х	US 5,726,195A (HILL et al.) 10 March 1998, see entire patent.	18-19
x	US 6,446,032 B1 (SCHIMMEL) 03 September 2002, see entire patent document.	20-21
X	WO 02/083837 A1 (PTC CORPORATION, INC.) 24 October 2002, see entire document, especially abstract, examples and claims.	18-21
Y	especiany austract, examples and claims.	1-24
x	WO 02/083953 A1 (PTC CORPORATION, INC.) 24 October 2002, see entire document,	18-21
Ÿ	especially abstract, examples and claims.	1-24
x	WO 01/25486 A1 (UNIVERSITY OF MEDICINE AND DENTISTRY OF NEW JERSEY)	18-21
 Y	12 April 2001, see entire document, especially abstract, examples and claims.	1-24
	documents are listed in the continuation of Box C. See patent family annex.	
	pecial categories of cited documents:  "T"  later document published after the interdate and not in conflict with the applicate and not in conflict with the applicate defining the general state of the art which is not considered to be  "T"  later document published after the interdate and not in conflict with the application of the art which is not considered to be	ation but cited to understand the
	defining the general state of the art which is not considered to be lar relevance  "X" decurrent of particular relevance; the c	
•	plication or patent published on or after the international filing date considered novel or cannot be consider when the document is taken alone	
establish t specified)	combined with one or more other such	when the document is documents, such combination
"O" document	referring to an oral disclosure, use, exhibition or other means being obvious to a person skilled in the	
priority da	published prior to the international filing date but later than the "&" document member of the same patent fate claimed	
	tual completion of the international search  Date of mailing of the international search  27 JAN 2005	h report
	2004 (13.12.2004)	
Mail Com P.O.	Stop PCT, Attn: ISA/US missioner for Patents Box 1450  Box 1450	5
	andria, Virginia 22313-1450 (703) 305-3230 Telephone No. 571-272-1600	

### INTERNATIONAL SEARCH REPORT

International application No. PCT/US04/09590

ategory *	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim N
X	GREER, C.L. Assembly of a tRNA Splicing Complex: Evidence for Concerted Excision and Joining Steps in Splicing In Vitro. Molecular and Cellular Biology. February 1986,	1, 7, 8, 11, 13, 1 and 17
Y	Vol. 6, No. 2, pages 635-644, see entire article, especially Fig. 1, pages 638-642.	1-24
Y	LI et al. Crystal Structure and Evolution of a Transfer RNA Splicing Enzyme. Science. 10 April 1998, Vol. 280, No.10, pages 279-284, see entire article.	1-24
Y	HYDE-DERUYSCHER et al Detection of Small-Molecule Enzyme Inhibitors With Peptides Isolated From Phage-Displayed Combinatorial Peptide Libraries. Chemistry & Biology. 2000, Vol. 7, No. 1, pages 17-25, see entire article, especially Abstract, Table 1 and pages 23-24.	1-24
A	ABELSON et al. tRNA Splicing. J. Biol. Chem. 22 May 1998, Vol. 273, No. 21, pages 12685-12688, see entire article.	1-24
A	TROTTA et al. The Yeast tRNA Splicing Endonuclease: A Tetrameric Enzyme With Two Active Site Subunits Homologous To The Archaeal tRNA Endonucleases. Cell. 13 June 1997, Vol. 89, pages 849-858, see entire article.	1-24
A	VAUGHAN et al. Methionine In and Out of Proteins: Targets for Drug Design. Current Medicinal Chemistry. 2002, Vol. 9, No. 3, pages 385-409, see entire article.	1-24
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International application No. PCT/US04/09590

#### BOX III. OBSERVATIONS WHERE UNITY OF INVENTION IS LACKING

This application contains the following inventions or groups of inventions which are not so linked as to form a single general inventive concept under PCT Rule 13.1. In order for all inventions to be examined, the appropriate additional examination fees must be paid.

Group I, claim(s) 1-3, 7(in part), 8, 11-14(in part), 15, 17(in part), 22 and 23-24(in part), drawn to assaying for an antifungal compound that inhibits fungal tRNA splicing ligase.

Group II, claim(s) 4-6, 7(in part), 9-10, 11-14(in part), 16, 17(in part) and 23-24 (in part) drawn to assaying for an anti-proliferative compound that inhibits animal tRNA splicing ligase.

Group III, claim(s) 18-19, drawn to preventing/treating a fungal infection by administering an antifungal compound identified by the Group I method.

Group IV, claim(s) 20-21, drawn to treating a proliferative disorder by administering an anti-proliferative compound identified by the Group II method.

The inventions listed as Groups I-IV do not relate to a single general inventive concept under PCT Rule 13.1 because, under PCT Rule 13.2, they lack the same or corresponding special technical features for the following reasons: the methods of Groups I and II are distinctly different methods drawn to different method objectives and utilize different sources of enzymes. The antifungal and antiproliferative compounds of Groups III and IV derived from the Group I and II methods do not represent a "special" technical feature since antifungal and antiproliferative compounds are known in the art. See e.g. see RNA binding "small molecule" compounds disclosed in WO 02/083953A1; WO 02/083837A1; and WO 01/25486A1.

Continuation of B. FIELDS SEARCHED Item 3: WEST: PGPB, USPT, USOC, EPAB, JPAB, DWPI, TDBD. STN: CAPLUS, EMBASE, BIOSIS, MEDLINE, WPIDS.